

Effectiveness Studies

Robert M. Kaplan

University of California, San Diego

NHLBI, NCI Research Phases

- hypothesis generation (phase I)
- method development (phase II)
- controlled intervention trials (phase III)
- studies in defined populations (phase IV)
- demonstration research (phase V).

Efficacy vs Effectiveness

- RCTs efficacy studies show that a treatment *can* work
- Evidence suggests that interventions are often less effective in clinical settings than in the laboratory (Weisz et al, 1992)
- Effectiveness trials evaluate treatments in the settings where they will be applied

Other Effectiveness Trials

- Community Wide Trials
 - SFCP
 - MHHP
 - PHHP
- Worksite Trials
 - WHP
- School Trials
 - CATCH

Community Wide Trials

- Stanford, Minnesota, Pawtucket
- Targeted changes in CHD risk factors
- None was randomized
- Communities matched
- Intervention length varied from 5-7 years

Results From Community Intervention Studies in CHD

- In Stanford, small effects for BP, cholesterol, smoking
- Few effects in Pawtucket and Minnesota
 - Minnesota showed reduction in smoking prevalence among women
 - Pawtucket showed smaller increases in body mass in intervention communities

Smoking Prevention Project

- 40 school districts randomly assigned to experimental or control groups
- Intervention included 15 “essential elements” identified by NCI advisory panel
- Students followed from grade 3 until 2 years after high school
- Result-- no long term benefit of intervention

Problems That Cloud the Interpretation of Effectiveness Studies

- Methodological
 - Loss to follow-up
 - Differential compliance
 - Uncontrolled influences
- Practical
 - Relapse is common
 - Long term behavior change is difficult to achieve

Approaches

- Statistical adjustments
 - Adjustment for differential dropouts
 - Imputations for missing data
 - “Worst case” assumptions
- Practical
 - Cohort maintenance (will be discussed on Friday)